

205T40" 6222550

Non-Transduced hMSCs in Immuno-competent mouse muscle

Day 3

DiI + hMSCs

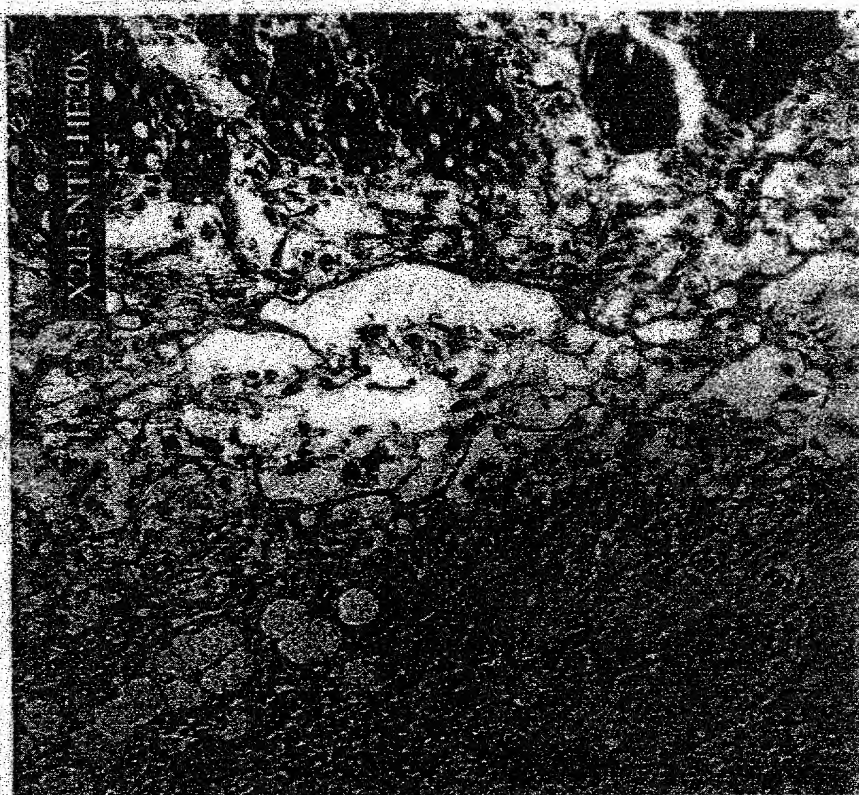
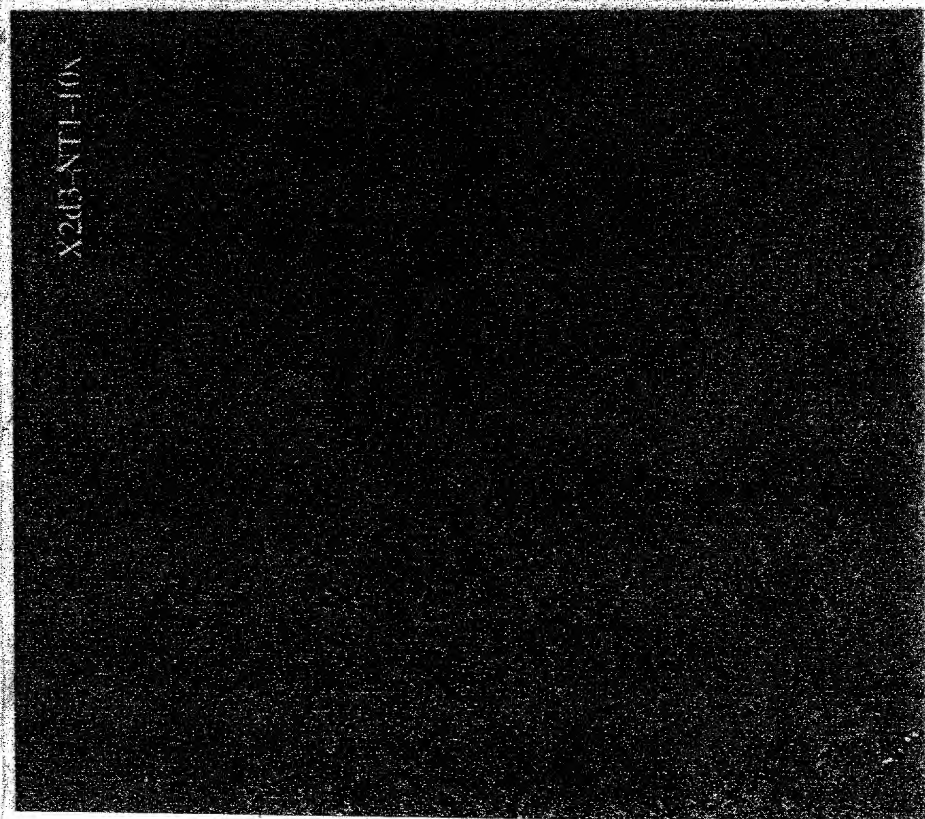


FIG. 1α

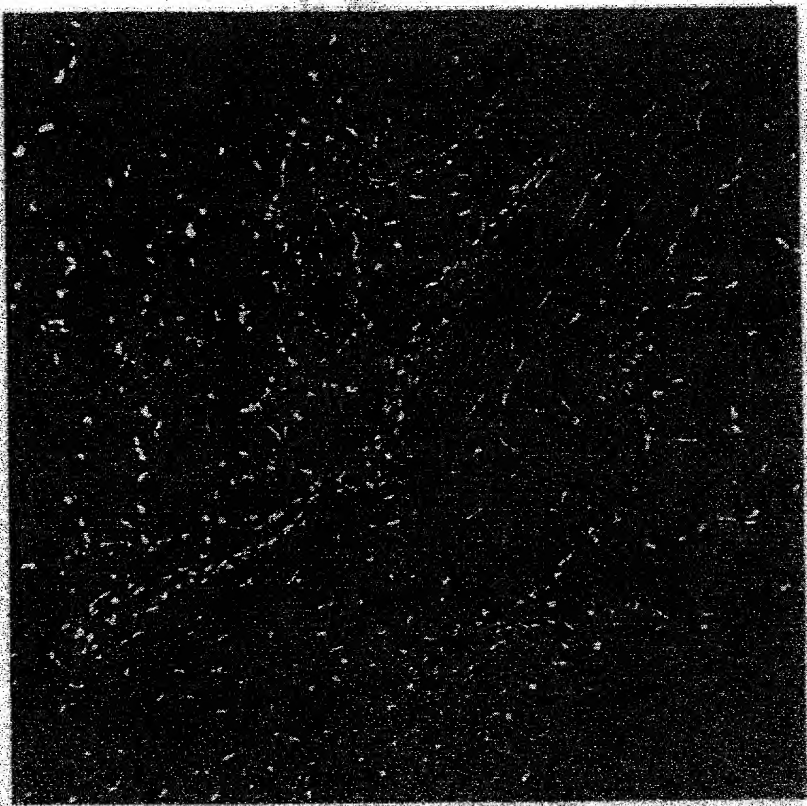
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# FIG. 1b

Human MSCs in Mouse Muscle: Day 7

DiI labelled cells +  
Sybr-green staining  
of all nuclei

(H&E)



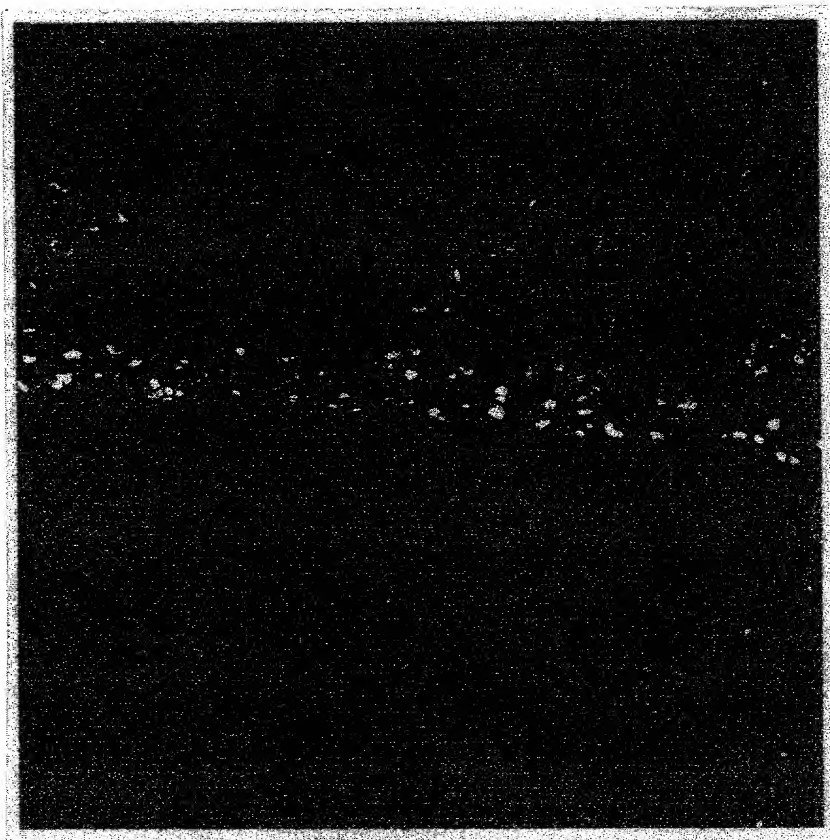
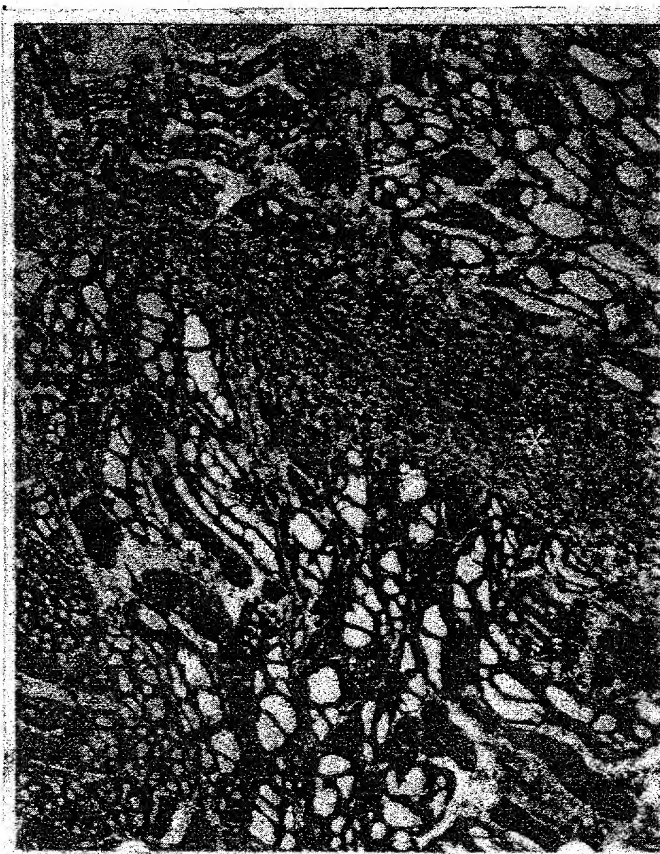
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# FIG. 1c

Human MSCs in Mouse Muscle: Day-7

(H&E, 20X)



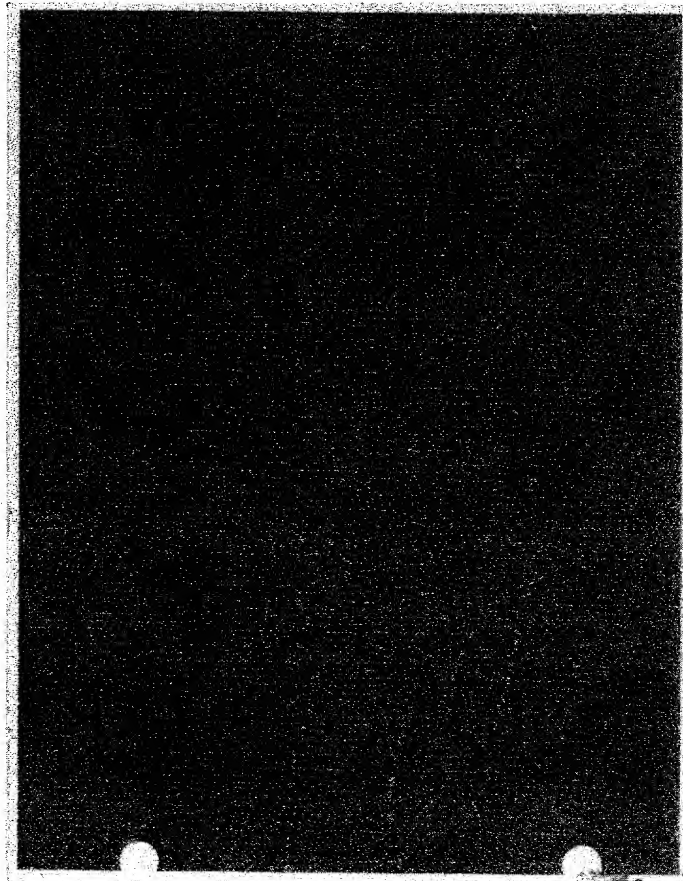
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# FIG. 1d

Human MSCs in Mouse Muscle: Day 14

Fluorescent Field, 10X



H&E 40X



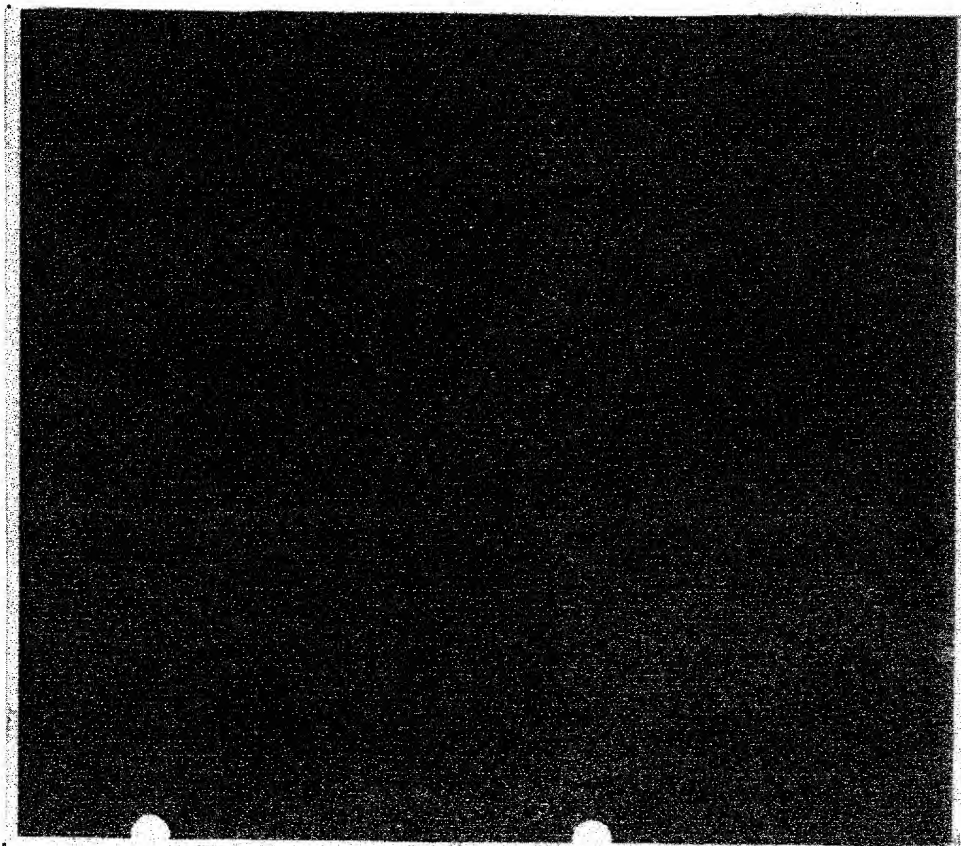
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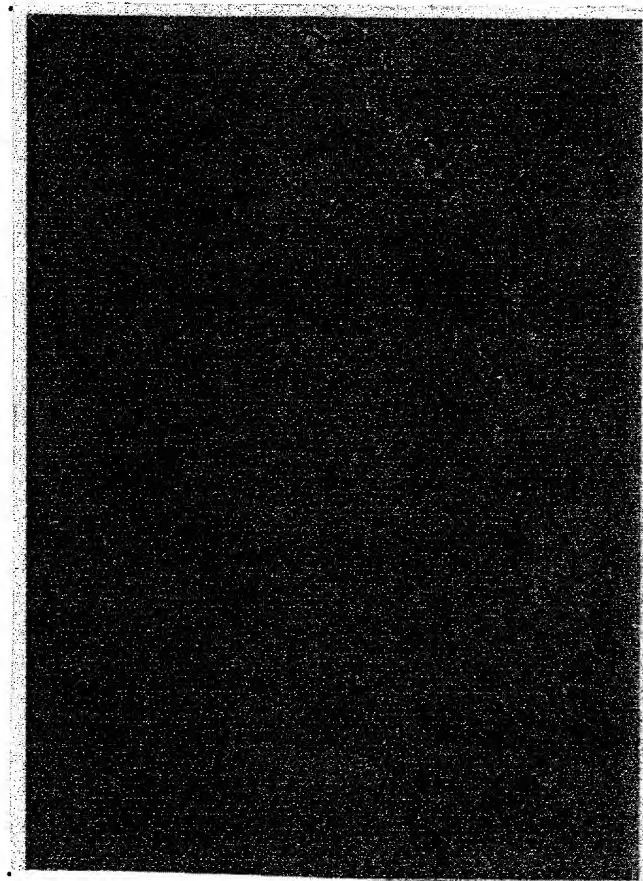
# FIG. 1e

DiI-Positive  $\alpha$ -Gala Transduced hMSCs in  
Immuno-competent mouse muscle

d29



d43



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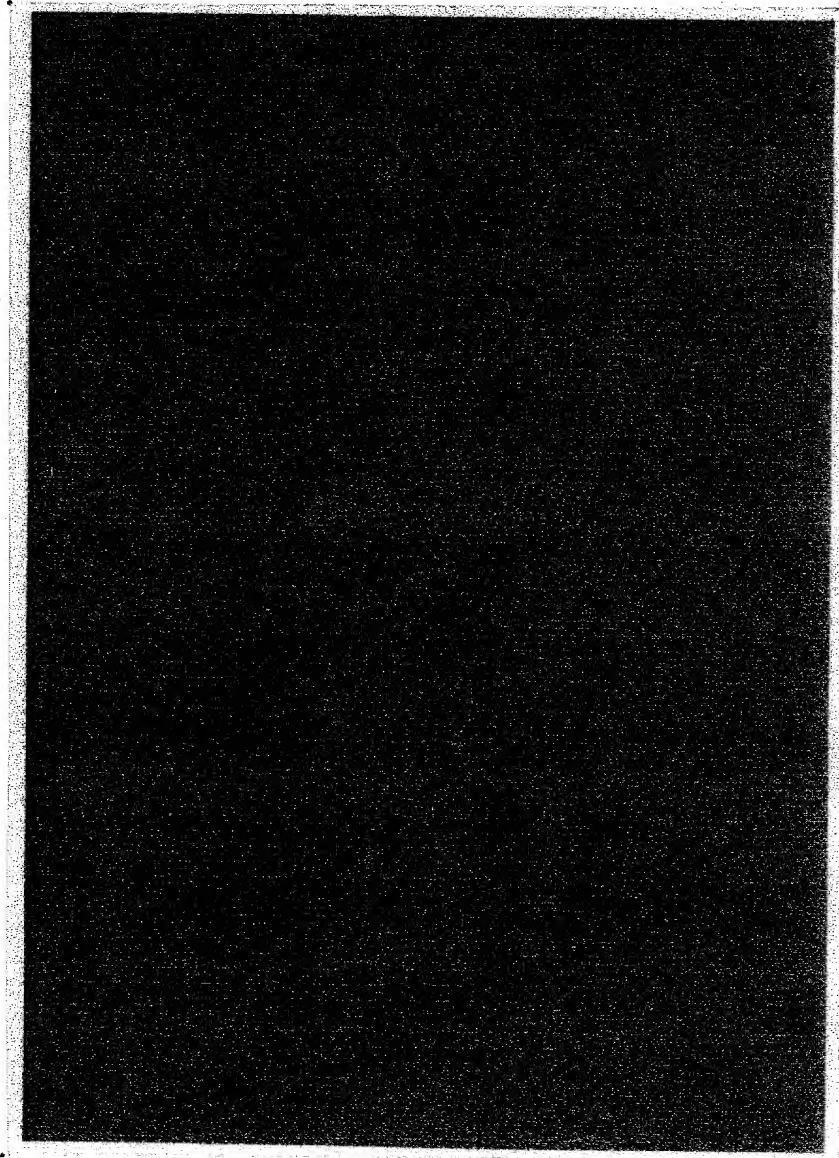
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# FIG. 1F

Human  $\alpha$  GalA-Transduced MSCs in Mouse Muscle: Day-57

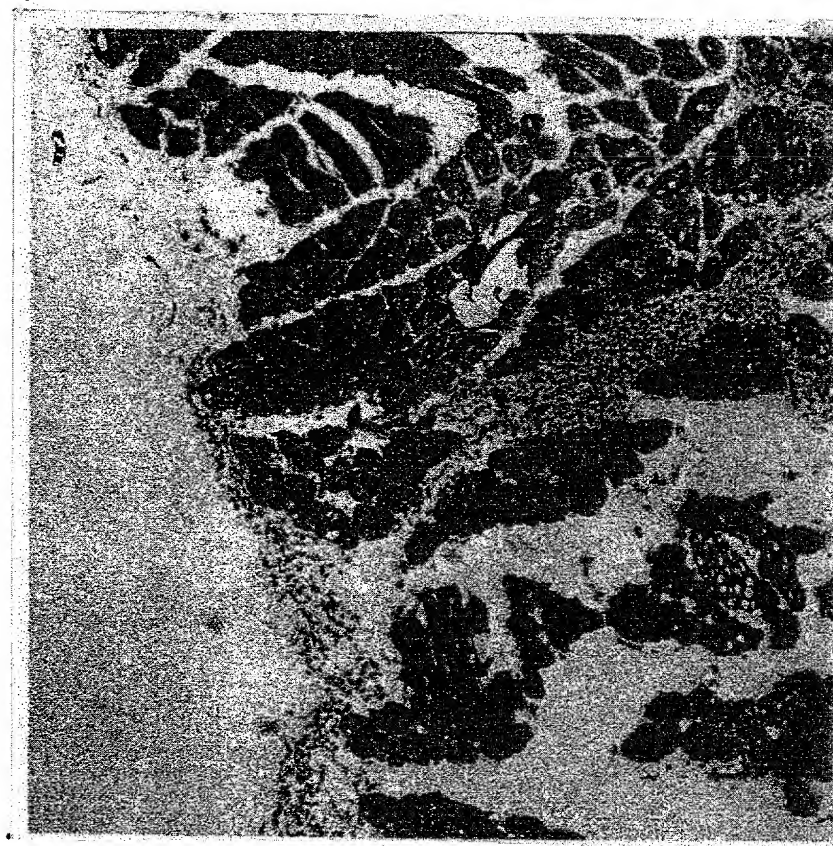
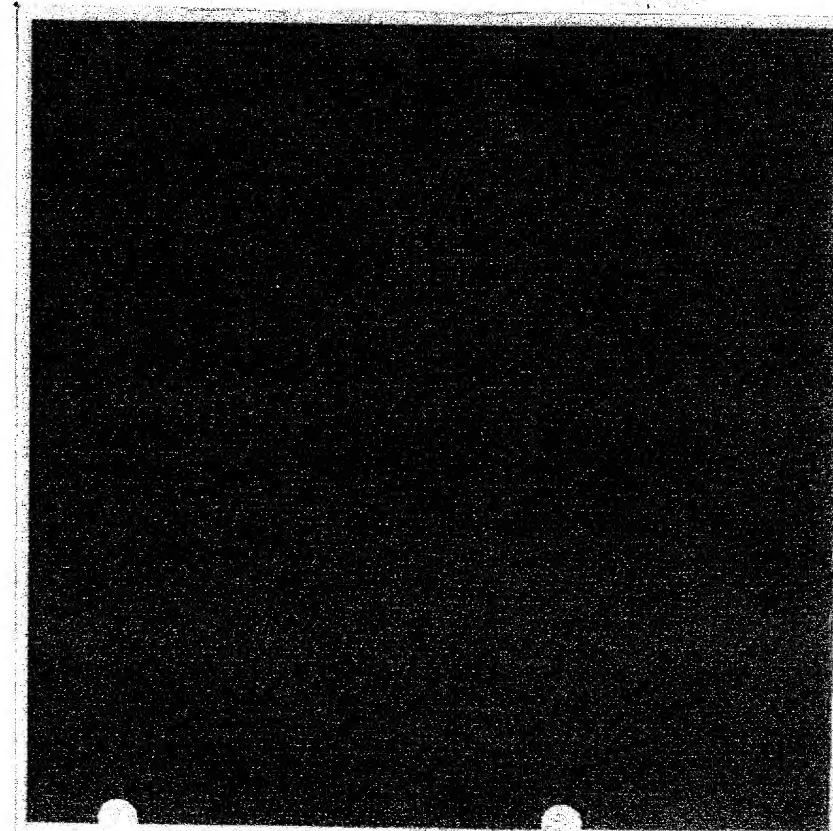
10X



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# FIG. 2

D3: IM injection of DiI-labeled PBMCs

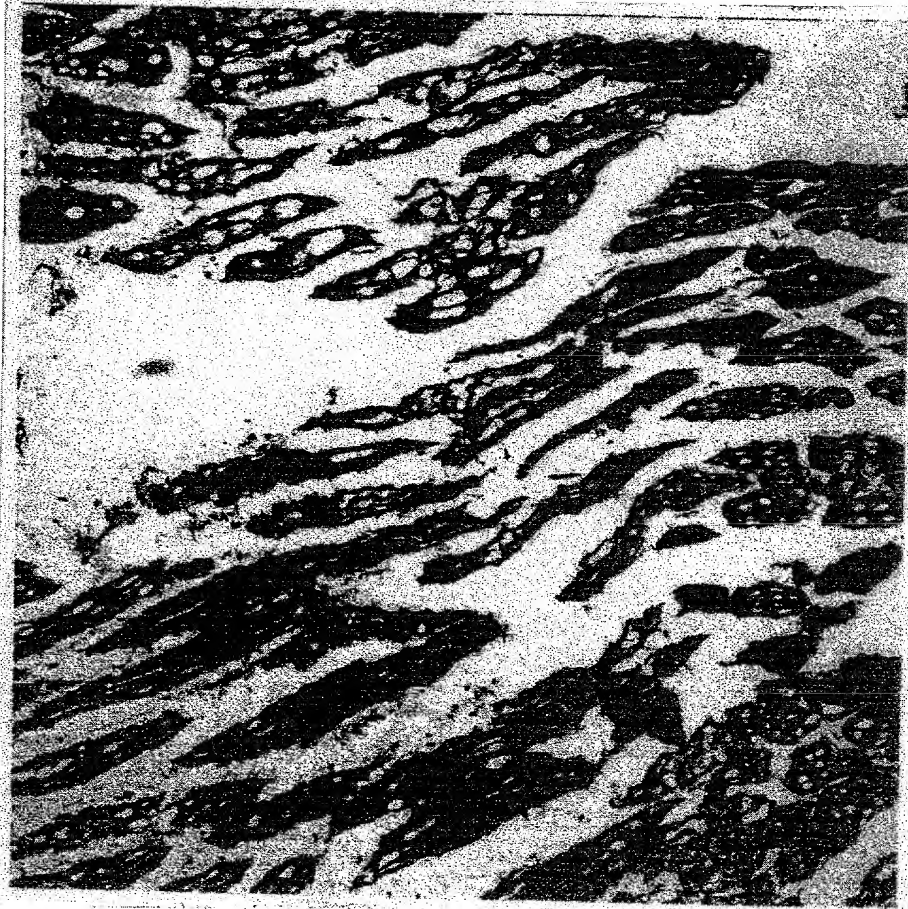


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# FIG. 3

D3 IM injection of saline

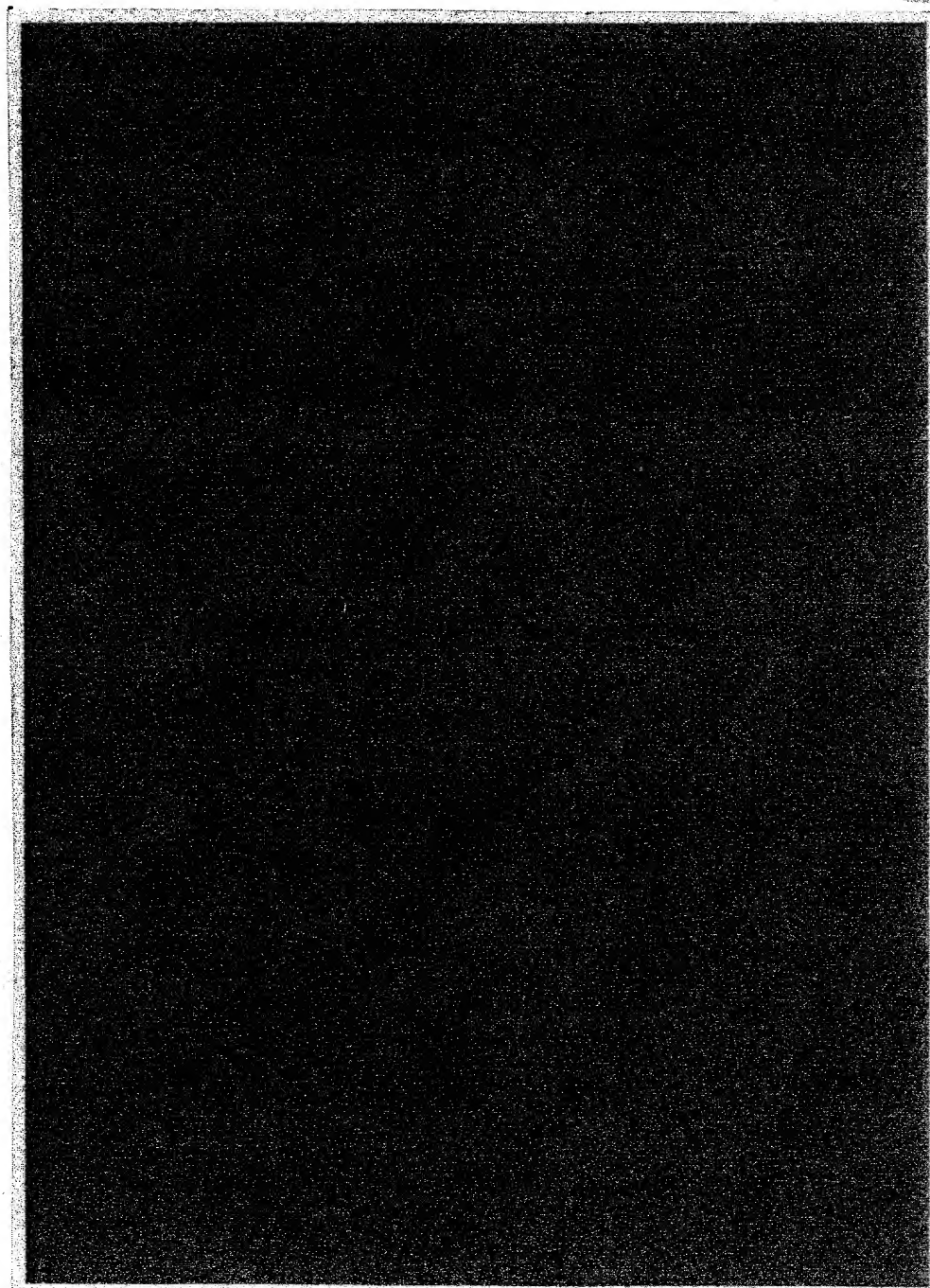


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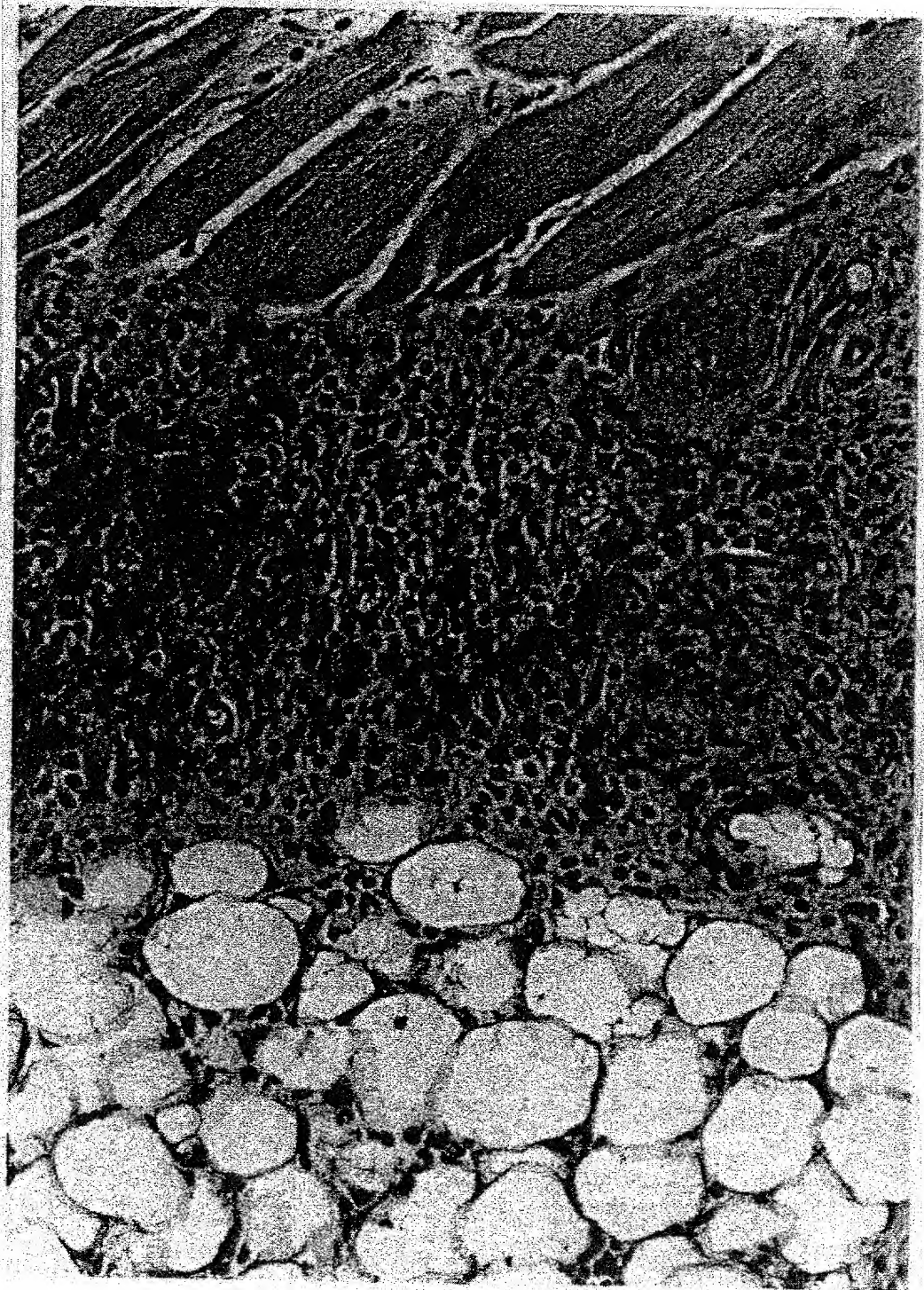
# FIG. 4

Red and Blue Florescence Channels merged



# FIG. 5

$\alpha$  GalA Immunostain of Muscle from Mouse  
injected IM with  $\alpha$ GalA-hMSCs: 14 days

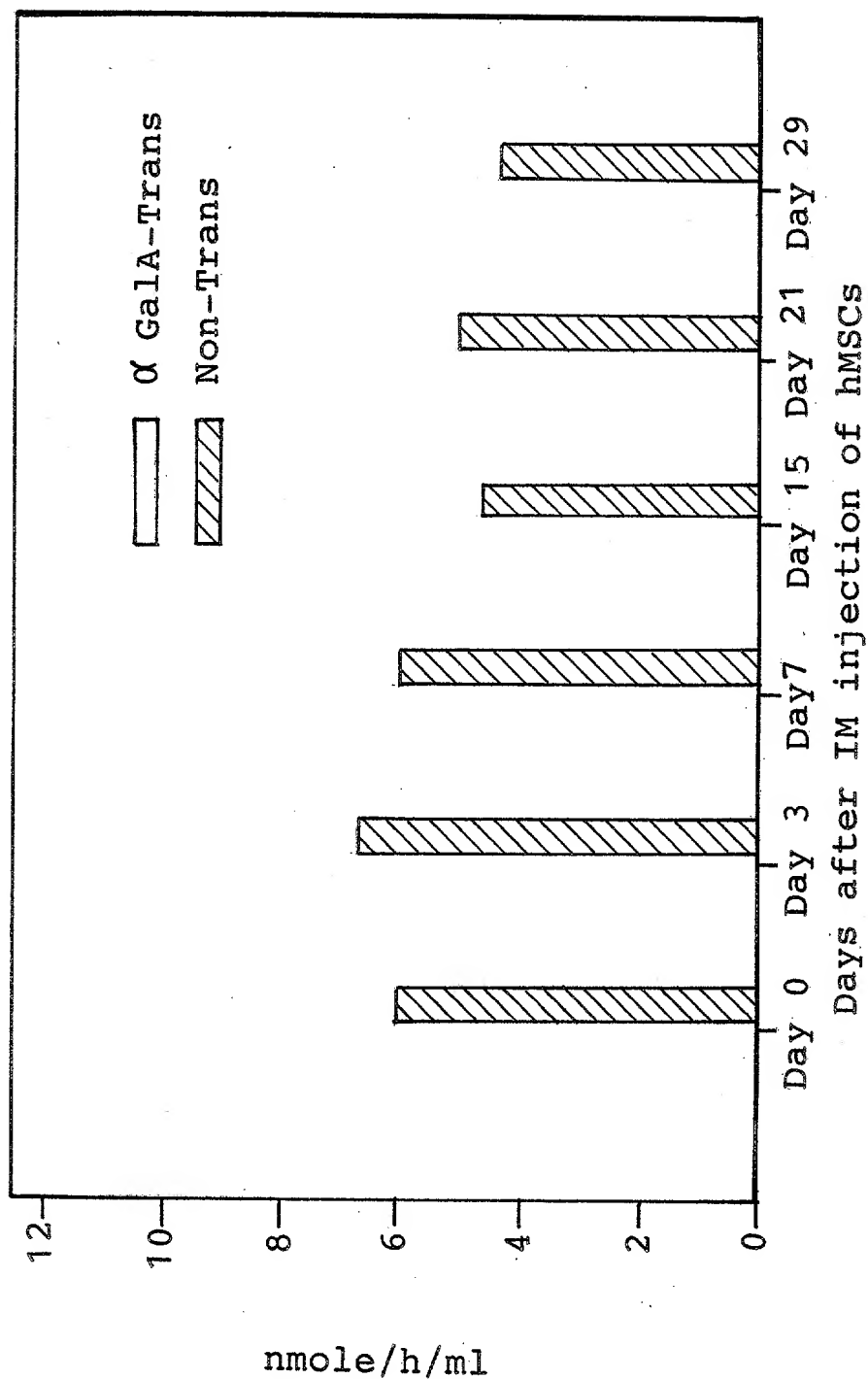


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## FIG. 6

$\alpha$ -Gala activity in Plasma of Mice  
IM-injected with  $\alpha$ -Gala-Transduced h MSCs



#: No MSCs, \*n=2,

Fabry Mice plasma: 0.5 nmole/h/ml, PNAS 97:7515

+

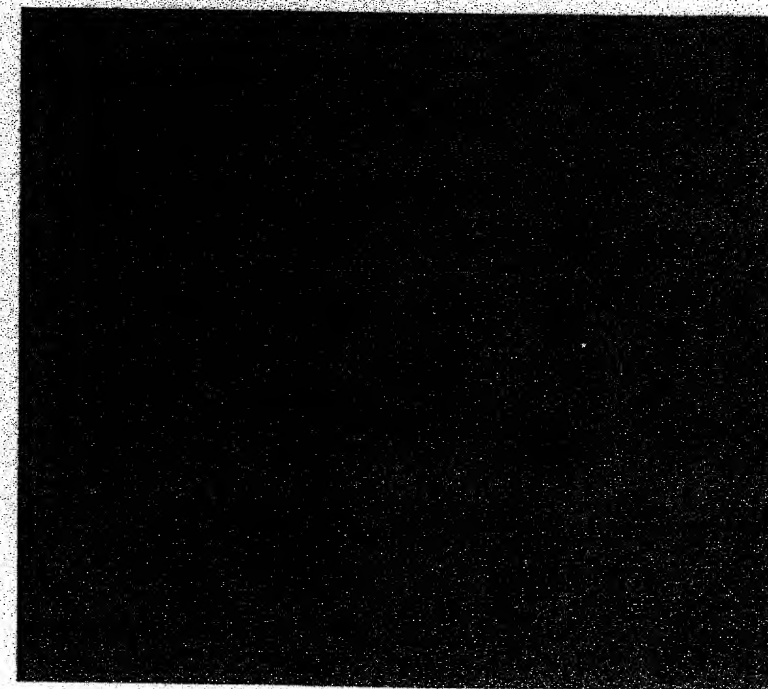
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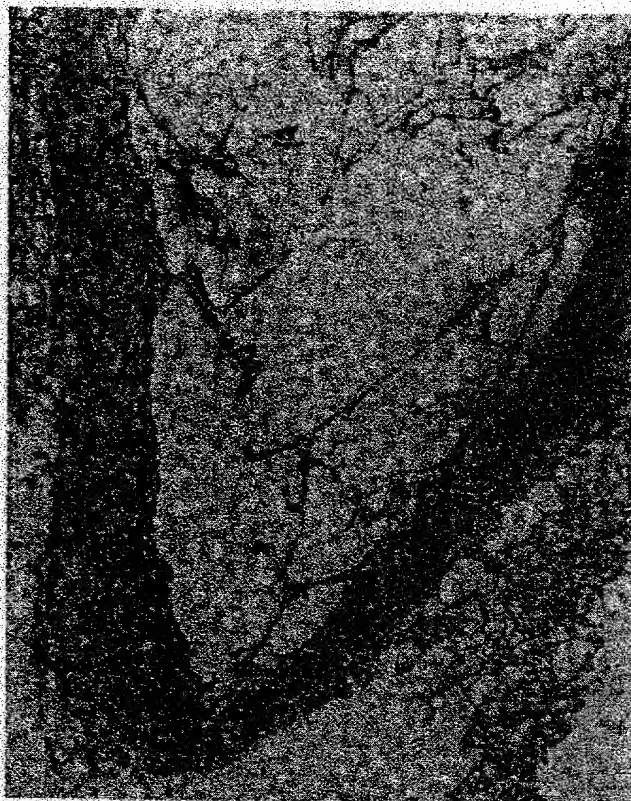
# FIG. 7α

Human-specific Alu esquence staining of hMSCs injected into Immuno-competent mouse muscle

**Dil-positive cells**



**Alu-sequence positive cells**

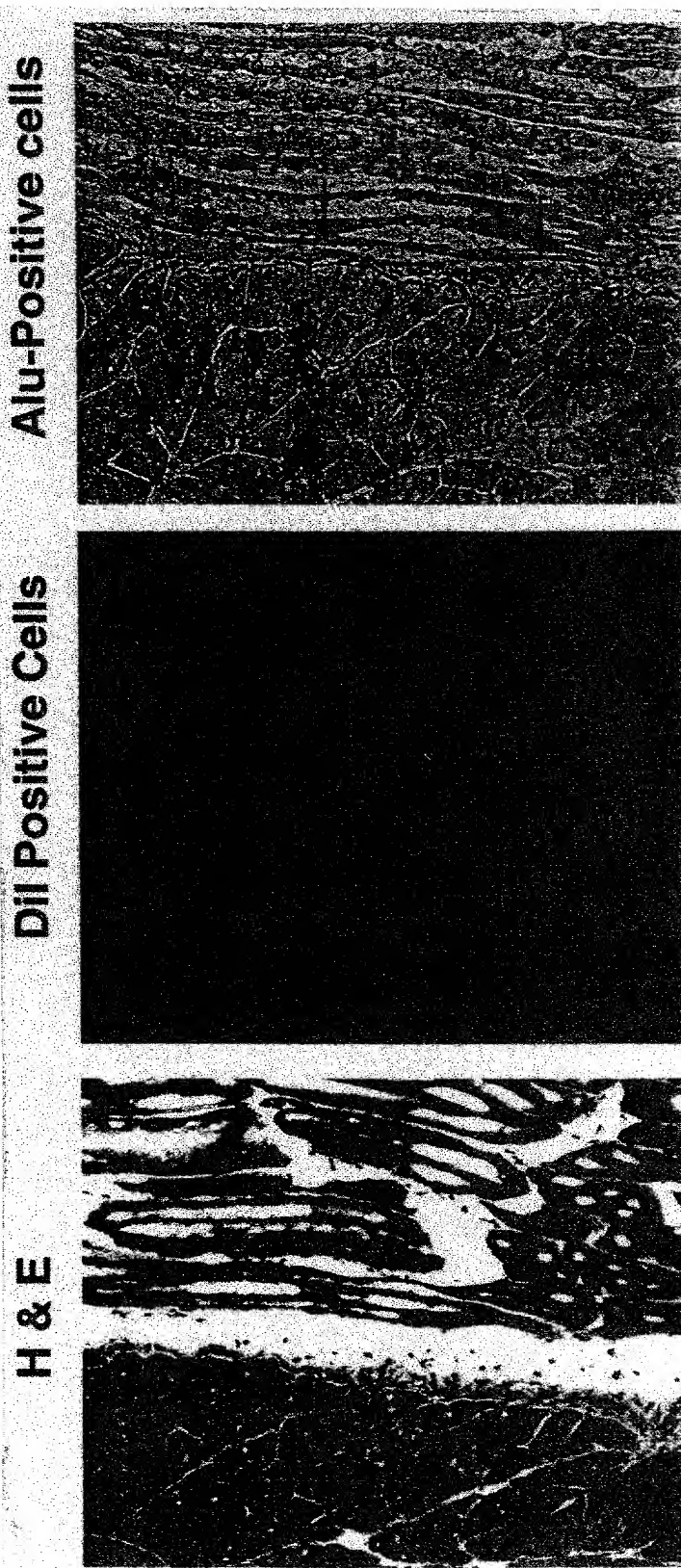


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# FIG. 7b

Human MSCs in Mouse Muscle: 43 days



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Day 57 DiI/Alu

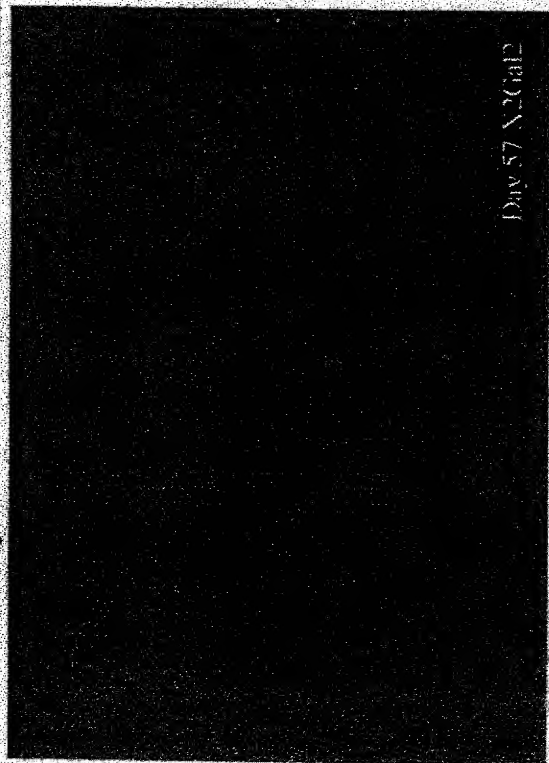


FIG. 7c

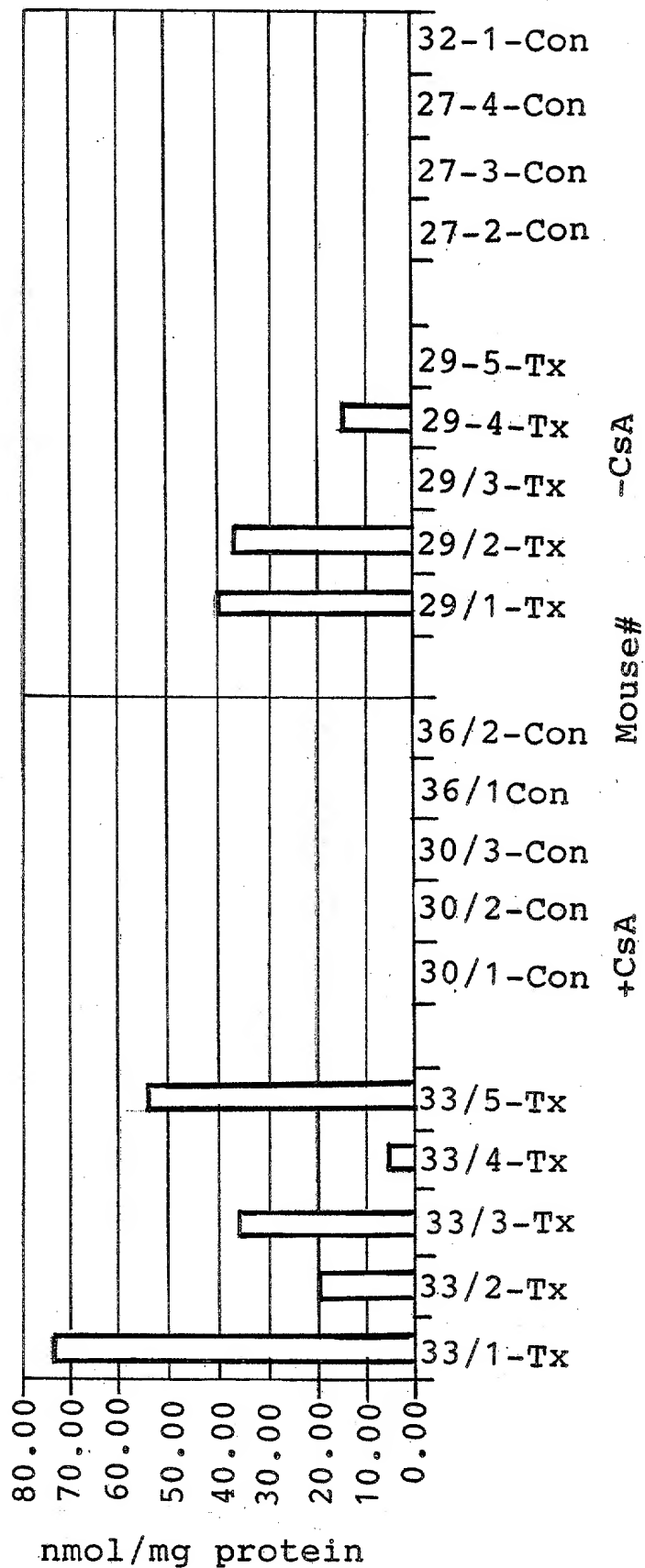
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F1 G. 8 $\alpha$ 

$\alpha$  Gala Activity in Muscles of Fabry-KO mice:  
14 days after IM injection of  $\alpha$  Gala-Transduced hMSCs

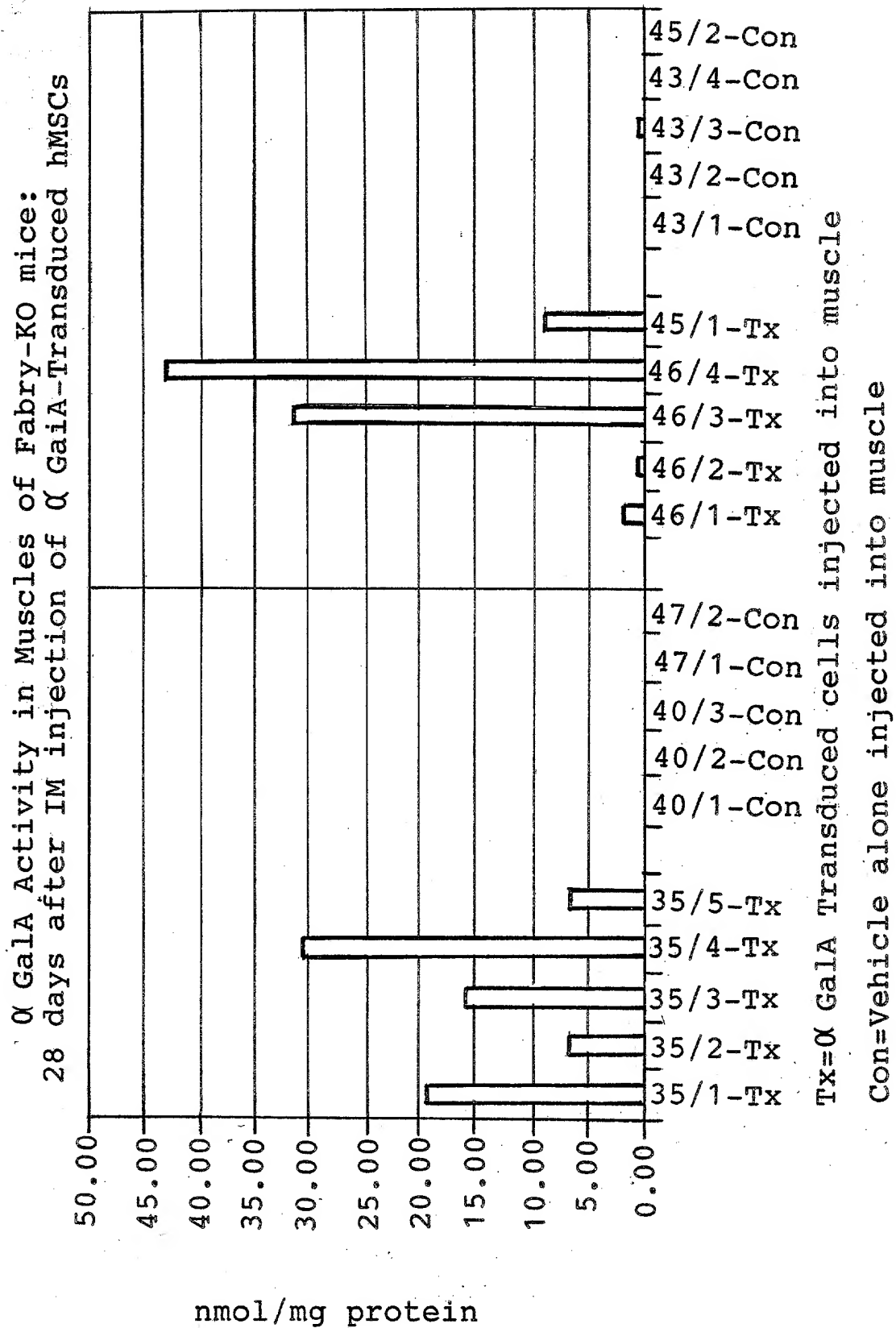


Tx =  $\alpha$  Gala Transduced cells injected into muscle

Con = Vehicle alone injected into muscle

+

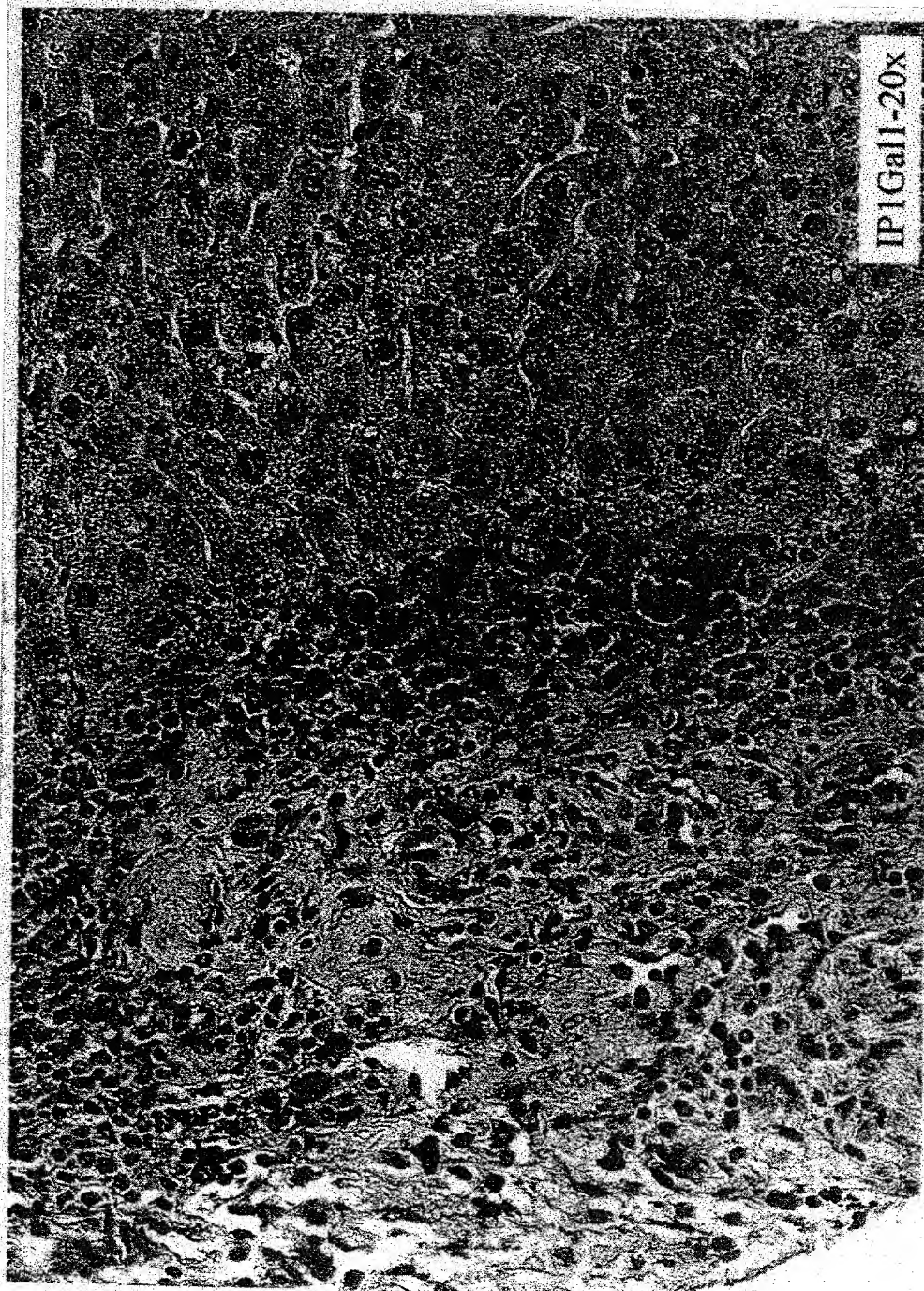
FIG. 8b



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FIG. 9a

$\alpha$ GAL4 Immunostain of Liver from KO Mouse  
injected IP with  $\alpha$ GAL4-hMSCs on Cultispheres:  
14 days



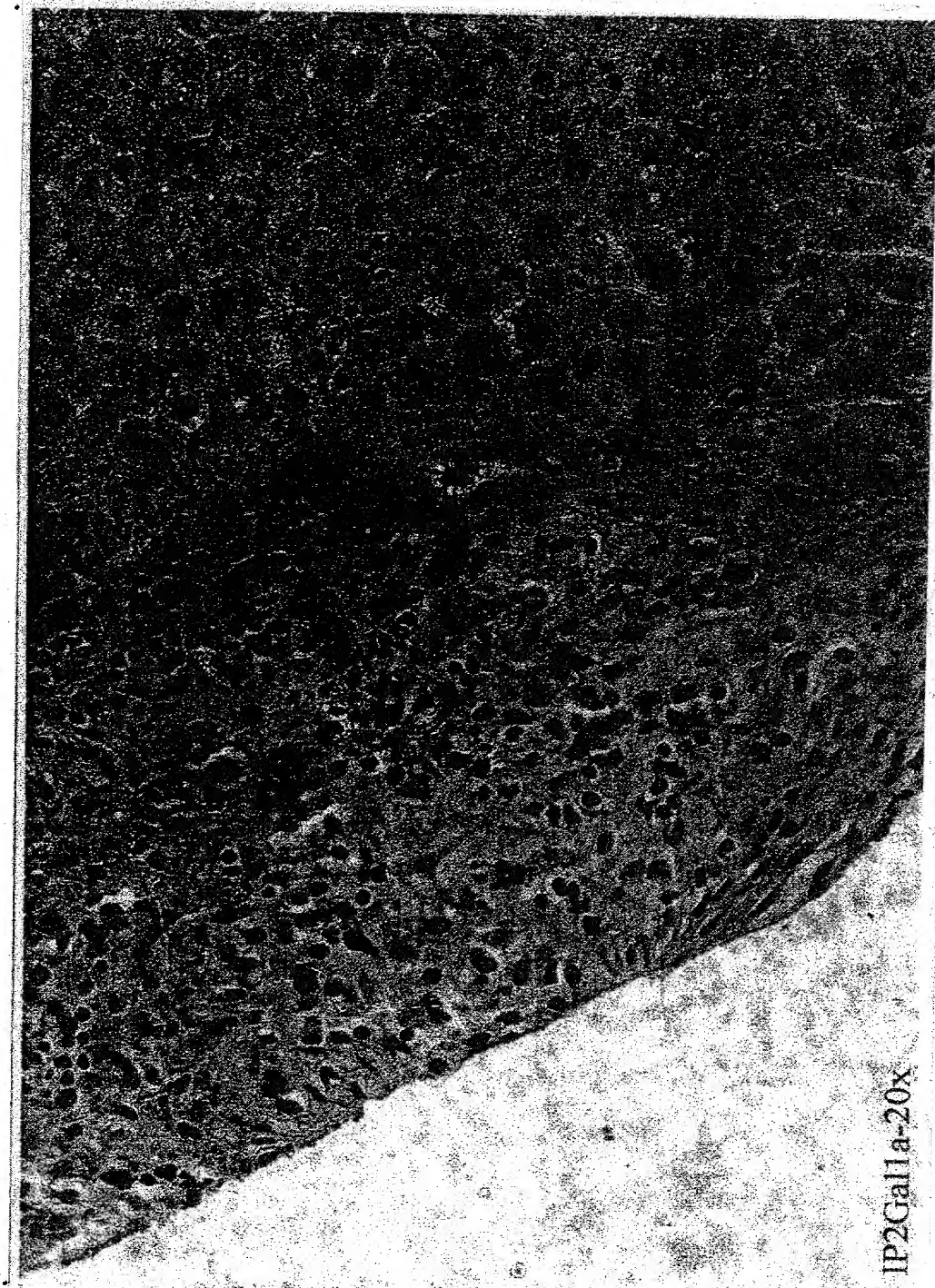
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# FIG. 9b

$\alpha$ GalA Immunostain of Liver from KO Mouse  
 injected IP with  $\alpha$ GalA-hMSCs on Cultispheres:  
 14 days



IP2Galla-20x

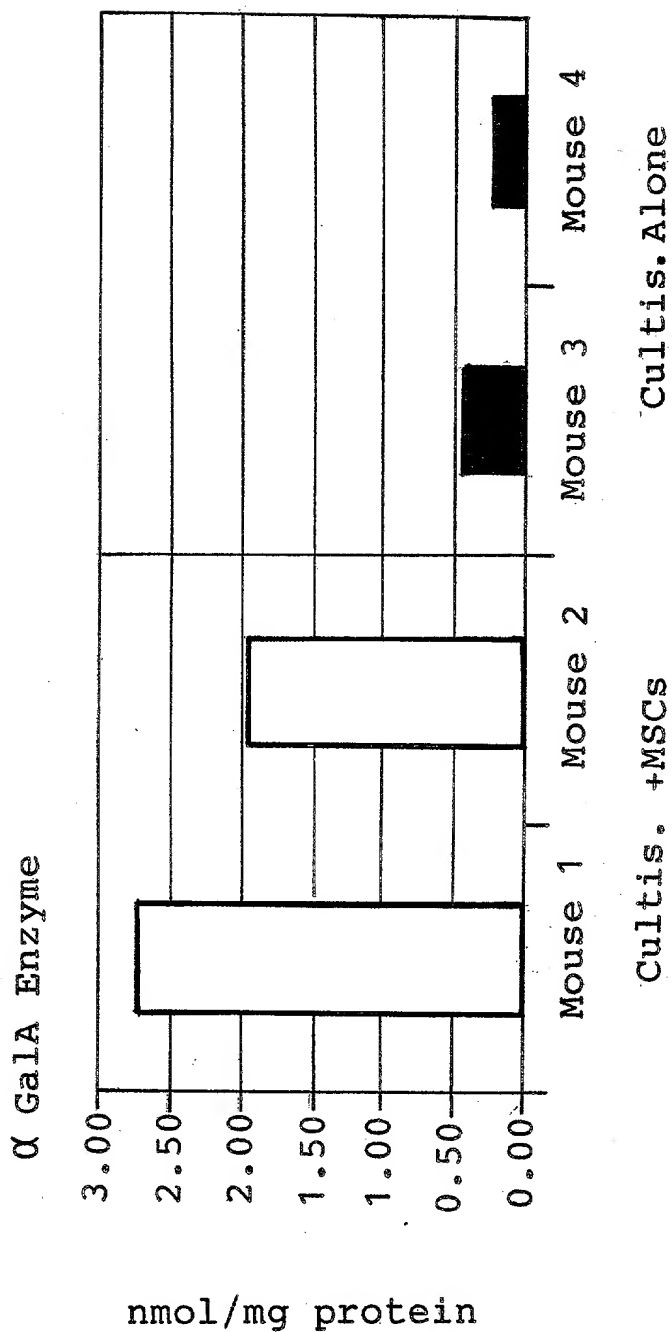
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# FIG. 10

Intra-Peritoneal Delivery of  $\alpha$  GalA-Transduced hMSCs  
to Fabry Knock-Out Mice



Increase in  $\alpha$  GalA is 5% of normal mouse liver activity (41 nmole/mg)

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